



July 16, 2018

The Honorable Alex Azar, Secretary
Department of Health and Human Services
Hubert H. Humphrey Building, Room 600E
200 Independence Avenue, SW
Washington, D.C. 20201

RE: Comment on CMS-2018-0075-0001 (“HHS Blueprint to Lower Drug Prices and Reduce Out-of-Pocket Costs.”)

Dear Secretary Azar,

On behalf of the 30 million Americans with one of the approximately 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) would like to thank the Department of Health and Human Services (HHS) for the opportunity to provide comments on the request for information titled, “HHS Blueprint to Lower Drug Prices and Reduce Out-of-Pocket Costs.”

NORD is a unique federation of voluntary health organizations dedicated to helping people with rare "orphan" diseases and assisting the organizations that serve them. Since 1983, we have been committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.

NORD strongly believes that all individuals with a rare disease should have access to quality and affordable health care that is best suited to their medical needs. Subsequently, we thank HHS for sharing our goal of increasing access to affordable treatment options. Far too many individuals with rare diseases and their families face exorbitant costs to maintain their health, and our community welcomes the potential to lower the cost of their healthcare while maintaining access to innovative healthcare options.

Consequently, we are pleased to provide comments to HHS on several of the policies discussed within the “HHS Blueprint to Lower Drug Prices and Reduce Out-of-Pocket Costs” (hereafter referred to as the “Blueprint”). We will review the policy considerations within the Blueprint using our guiding principle of increasing access to affordable innovative therapies for the rare disease patient population. We will also provide additional viewpoints on a handful of policy items not directly addressed by the Blueprint.

While our comments are not exhaustive, they capture our advice and guidance on how to proceed in a patient-focused manner on several of the policy proposals.

Increasing Competition:

We thank HHS for exploring methods to increase competition in the marketplace between Food and Drug Administration (FDA)-approved innovator and generic/biosimilar products. Greater competition, while simultaneously avoiding disincentivizing innovative orphan drug development, also results in greater choice for patients between alternative products.

Unfortunately for those in the rare disease community, there are rarely generic or biosimilar products for orphan therapies due to the small patient populations that would take such generic or biosimilar therapeutics. Even when generics do enter the market, evidence shows that it takes at least two generics to substantially lower costs for patients.¹ We recommend that HHS consider policies that could spur the development of generic and biosimilar orphan products in order to increase affordable options for our community.

Educating Providers and Physicians. We at NORD are greatly in favor of increasing efforts to educate patients and providers on biosimilars. Very few rare disease patients have a treatment that has been approved by the FDA and is indicated to treat their condition. Even fewer rare disease patients have access to treatments that have less expensive therapeutically equivalent alternatives. Consequently, NORD is supportive of policies aimed at increasing the development of, and access to, biosimilars.

In general, the lack of biosimilar orphan products on the market also leads to continued misunderstanding of what biosimilars are within our community. We thank FDA for its efforts to educate the public through the Biosimilars Education and Outreach Campaign, and we look forward to further educational and outreach efforts from FDA to continue to publicize biosimilars and their characteristics. NORD will also continue educational efforts for our community, and would welcome the opportunity to partner with FDA on these initiatives.

Further, in our work in the states, we frequently advocate for legislation that requires pharmacists to communicate all dispensations of a biosimilar to the patient and the prescribing physician. The intent of this legislation is to ensure that providers are in the best position possible to care for their patients, and patients are not inadvertently harmed by an alteration in their course of treatment. NORD encourages HHS to consider this concept in its efforts to improve access to biosimilars through better understanding of their development and appropriate use.

Better Negotiation:

NORD agrees with HHS that there are substantial inefficiencies and opportunities for improvement within the therapeutic delivery system. We applaud HHS for seeking ways in which to improve this opaque and convoluted structure, but we unequivocally urge HHS to consider the effects these proposals may have on patients as it considers new pathways forward.

¹ Dave CV, Hartzema A, Kesselheim AS. Prices of generic drugs associated with numbers of manufacturers. *N Engl J Med.* 2017;377(26):2597-2598.

Many of the proposals within this section could be beneficial to our patients, but only if the policies pursued are patient-focused. For example, enhanced negotiations between drug delivery stakeholders may be beneficial, but only if it does not occur at the expense of patient access. These negotiations must also be transparent for patients to understand and digest.

Value-Based Arrangements and Price Reporting. Value-based pricing arrangements hold the promise of aligning prices and costs with the value the product brings to the patient. This ideally would allow for innovative safe and effective products to be rewarded for the health improvements they bring while ensuring their price is aligned with their value.

Unfortunately, value-based pricing arrangements too often define “value” as the greatest value for the payer’s (both private and public) budget without taking any patient viewpoints into account. What might be the cheapest for the insurer may not be of much value for the beneficiary, and could even worsen their health due to lack of access to alternatives.

We urge HHS to craft value-based pricing arrangements that defines “value” using patient viewpoints. This not only includes clinical improvements that orphan products can bring to patients with a rare disease, but also quality-of-life improvements for them and their loved ones. For example, a new orphan product for a child may allow the parents to sleep through the night, or pursue employment as they no longer need to be a full-time caretaker. In addition, certain rare diseases, if caught early, are largely treatable and do not result in substantial long-term healthcare needs. Additional values that should be captured include values to friends and families based upon the improved health of their loved one, as well as societal values deriving from the improved health of an individual with a rare disease.

These values can only be captured by engaging the patient communities who live these realities every day. The patient community is ready to participate in such conversations just as we have participated within FDA opportunities to better include patient viewpoints in drug development.

NORD in particular is eager to facilitate such agreements by serving as the patient intermediary in value-based pricing arrangements. In coordination and collaboration with the specific patient community intended to receive the therapy, NORD can ensure value-based pricing arrangements are patient-friendly and patient-focused, and can help craft agreements that encourage innovation while aligning costs with value. We’re excited for this opportunity, and we’re hopeful to serve as a resource as HHS further explores these possibilities.

Indication-Based Payments. Similar to other forms of value-based pricing, indication-based payments can be beneficial if the pricing is aligned with the value the therapy brings to the patient. If the payment for the specific orphan indication reflects the values the therapy brings to the patient, these arrangements can help incent the pharmaceutical industry to develop secondary uses or indications for rare disease populations that could benefit from existing therapies.

However, once again, indication-based pricing used recklessly and without patient involvement could inaccurately assess the value derived from these products by particular patient populations captured in certain indications. This inaccurate assessment could lead to a lack of access to medically-necessary care.

In addition, indication-based pricing may not account for the over 80 percent of rare disease patients who rely on therapies taken off-label. Approximately 95 percent of rare disease patients still do not have an FDA-approved treatment for their specific disease. Instead, they rely on off-label therapies to treat their symptoms. If indication-based payments particularly disadvantage off-label uses due to lack of data to support these uses, the rare disease patient population would be grossly disproportionately harmed. We urge HHS to consider ensuring off-label access as it evaluates indication-based payment models.

Within this section, HHS asks, “How and by whom should value be determined??” We hope HHS agrees that value should be determined by those we all serve; the patients.

Long-Term Financing Models. We currently find ourselves on the precipice of unparalleled medical innovation in the form of gene editing and gene therapy technologies. These scientific breakthroughs represent the opportunity to cure chronic and debilitating rare diseases, and allow children and adults with rare diseases the opportunity to lead healthy lives.

Due to the unique nature of these high-cost, often one-time curative therapies, innovative payment and financing models are needed in order to appropriately reward innovation while ensuring health financing stability. While these therapies present unique challenges, we do not believe them to be insurmountable. But we do face urgency in ensuring these therapies that are already starting to come to market can be accessed by the patients who need them.

Consequently, NORD strongly urges HHS to expedite its efforts to facilitate innovative payment and financing agreements between curative-therapy developers and public and private payers. There are various ideas to consider, including tying payments to long-term value (once again defined by the patient) of the potentially curative therapy, amortizing the payments for the therapy over time to avoid unrealistic one-time payments, and facilitating the responsibility of the payments to be portable across private and public insurers to avoid adverse selection concerns. Other proposals have included re-insurance programs, or other means to backstop insurers who may face paying for a particularly-expensive cure.

These challenges, while daunting, can be overcome. We welcome the opportunity to work with HHS and all stakeholders in considering innovative financing models in order to ensure our patients are able to obtain these miracle cures.

Part B to D. NORD understands that the Medicare Part D program offers additional opportunities to lower the costs of certain medications that the Medicare Part B program does not afford. However, we have strong concerns that moving therapies from Part B to D may have a substantial detrimental impact on our patients who rely on these therapies.

First, several empirical analyses have concluded that moving therapies from Part B to Part D will result in higher out-of-pocket costs for the patients that rely on these therapies. For example, a 2011 study commissioned by the Center for Medicare and Medicaid Services (CMS) found that patients whose therapies were moved from Part B to D faced substantially higher out-of-pocket

costs.² Many Medicare beneficiaries with a rare disease simply cannot afford the additional hundreds of dollars they would be required to pay if their therapy is moved from Part B to D.

Second, Part D plans are permitted to employ various utilization management techniques such as step therapy and prior authorization requirements as well as specialty tiers with high co-insurance. While often intended to encourage beneficiaries to explore lower-cost alternatives to expensive therapies, these tools are often used when there are no therapeutically-equivalent therapies, or even no alternatives whatsoever. Therefore, these utilization management tools can purely punish a rare disease patient for requiring the only medically-necessary therapy available to them, potentially resulting in adverse health impacts due to lower adherence.

Finally, we are concerned that moving therapies from Part B to D could result in substantially higher premiums for Part D beneficiaries, further increasing costs for our patients. This would cause the Part D program to become less affordable, potentially causing some older Americans to no longer seek care and suffer adverse health impacts as a result.

We applaud HHS for seeking ways to increase innovation in how Medicare pays for therapies and covers patients with rare diseases. But such innovation should not come at the expense of our patients, and we urge HHS to carefully consider the impacts these changes could have on vulnerable Medicare beneficiaries.

Create Incentives to Lower List Prices:

Once again, we thank HHS for tackling the complex and convoluted drug delivery pathway that can too often incent all players to pursue higher list prices. The actors that each play a role, including the drug developer, the pharmacy benefit manager (PBM), the specialty pharmacy (in the case of the many orphan therapies), and the insurer all must similarly be encouraged to keep costs to the patient low.

Reducing the Impact of Rebates. NORD supports addressing the current rebate structure as it pertains to the Medicare program with the goal of addressing perverse incentives to raise list prices. The current system in which PBMs receive a certain percentage of the rebate negotiated with the manufacturer does not help patients receive lower-cost therapies. In fact, not only may this incentivize manufacturers to raise their prices, but the co-insurance patients pay is usually based upon the original list price, not the negotiated price and accompanying rebates.

We encourage HHS to be particularly careful as it considers policy options to address this issue. Too often we have seen well-meaning policy interventions that attempt to lower costs for patients only raise their costs elsewhere. We ask HHS to ensure that whatever intervention is pursued does not allow the insurer to raise costs elsewhere to compensate for the lost revenue.

Copay Discount Cards. Third-party copay, coinsurance, and premium assistance represents one of the most effective means for ensuring patients receive the medically-necessary

² “Estimating the Effects of Consolidating Drugs under Part D or Part B”, Acumen, LLC, found at: https://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/Reports/downloads/Acumen_B_to_D_Final_Report_2011.pdf

care they require. Many individuals with a rare disease rely on third party assistance to access expensive therapies, and without such assistance, adherence to safe and effective treatment options may lower significantly.

We understand that many are concerned that copay discount cards shield the true cost of a therapy from the beneficiary, and eliminate any incentive for the patient to choose lower cost treatment options instead. However, within rare diseases, it is much more likely that there is no alternative for the orphan drug the patient is taking, and the alternative is to simply not have any treatment options at all. Further, patients and families affected by rare disease are more often in financial distress and will not be able to afford their copays if the majority of the cost is transferred to them. Those without insurance will not be able to afford it at all.

Consequently, we reject the argument that copay cards or other forms of third party assistance artificially inflate demand for orphan therapies without an alternative. Instead, these programs may allow patients who otherwise could not afford to take these medically necessary therapies the chance at a healthier life. We would also welcome the opportunity to collect and disseminate empirical analysis that quantifiably illustrates our position.

NORD is the administrator of various orphan drug copay assistance programs for patients in Federal health programs. Before commenting on whether or not to allow manufacturer copay assistance programs to expand into Federal health programs, additional reasoning from HHS would be helpful for us to understand what inadequacies HHS sees within the current third-party assistance paradigm involving charity administrators, and why manufacturer copay assistance programs within Federal health programs would be advantageous comparatively.

Federal Preemption of Contracted Pharmacy Gag Clauses. NORD believes that the existence of the pharmacy gag clauses provides no benefit to our patients. Individuals with rare diseases should always have the lowest-cost option available to them, and such gag clauses appear to combat this availability.

This being said, purchasing a therapy outside of insurance coverage would not allow the payment to count towards the deductible or the out-of-pocket maximum. Therefore, we can imagine scenarios in which patients may choose to pay less for their therapy outside of their insurance, but consequently pay more overall for their healthcare as they take longer to exhaust their deductible and reach their out-of-pocket maximum.

We ask HHS to carefully consider these scenarios before allowing, or at least encouraging, patients to explore purchasing their therapies outside of their insurance plan.

Medicare Part D Plan Flexibility and the Six Protected Classes

While not directly addressed in any HHS proposal, offering Medicare Part D plans additional structural and design flexibility, including with the six protected classes, is alluded to at various times. We understand the motivation to offer Part D plans additional flexibility to further

incentivize the use of lower-cost therapies, but too often we find insurers creating additional barriers to access in the name of flexibility.

For example, without the proper oversight, insurers implement step therapy requirements and incredibly arduous co-insurance requirements for drugs that do not have a therapeutically-equivalent alternative.

Similarly, if the six protected classes are amended, we would imagine many therapies that our patients rely on would likely no longer be covered. Flexibility for plans and incentives for manufacturers should not come before patient access; the protected classes and their impact on patients is too important to risk for the slight, hypothetical chance of lower prices.

Orphan Drugs and the Orphan Drug Act

As the organization that successfully advocated for the passage of the Orphan Drug Act (ODA) 35 years ago, NORD continues to unequivocally support the ODA and the incentives within. Nowhere within the Blueprint does HHS propose any changes to the ODA, and thank HHS for this position.

However, there are others who may ask HHS within their comments to change the ODA. We want to ensure that HHS understands that NORD does not support any changes to the ODA at this time as we have not seen any empirical data that would indicate reforms are needed. We have convened an Orphan Drug Task Force to investigate many of the accusations of abuse that have been leveled at the ODA, as well as craft potential improvements to the ODA. As the Orphan Drug Task Force continues to deliberate, we will ensure HHS has all relevant outcomes.

In the meantime, we ask HHS to stand with the rare disease patient community and reject all harmful changes to the ODA. In addition, we also ask HHS to carefully consider how the other policies for which it may pursue will affect the development of innovative orphan therapies. The orphan drug development system, while successful, is delicate, and can be easily disrupted by changes elsewhere within the ecosystem. We ask that HHS consider this situation carefully as it moves forward with any of the proposed changes within the Blueprint.

NORD thanks HHS once again for the opportunity to comment. We look forward to working with HHS to ensure that rare disease patients will have access to quality and affordable therapies. For questions regarding NORD or the above comments, please contact me at pmelmeyer@rarediseases.org or 202-545-3828.

Sincerely,



Paul Melmeyer
Director of Federal Policy